



Clinical Research Trials: The Basics

A clinical research trial tests if something, like a new medication or medical device, is safe and effective for people to use. There are clinical research trials for most types and stages of cancer. Clinical trials are designed to find out how well a treatment works. Studies may be done on a worldwide level (at places of care around the world), national level (at places around the country), or at a single place of care. Studies can be set up to treat a certain stage or type of cancer.

The clinical trial may be the first treatment you get, or it could be an option after other treatments did not work. Trials can also test how medicines and other treatments may help prevent cancer, ways to find cancer earlier, or ways to help manage side effects. This article will help you understand the basics of clinical trials and how important they are in the field of oncology.

Oversight of Trials

An Institutional Review Board (IRB) is a group of healthcare providers, researchers, and people who do not have a background in medicine. The IRB's job is to:

- Review and approve any clinical trial before it can begin.
- Make sure the study is fair, properly designed, and safe for the patients.
- Once the study starts, the IRB:
 - Watches for safety issues.
 - Makes sure that researchers are sticking to the study plan.
 - Watches for anything that may affect the people in the study.

Informed Consent

If you are thinking about joining a clinical research trial, you must be fully informed about the trial details, benefits, and risks. This is explained during a process called informed consent. The study team and provider must explain the trial in a way that you and your caretakers can understand. You should be able to have all of your questions answered. This helps you and your loved ones make an informed decision about whether to take part in the trial.

You must sign an informed consent form to take part in a trial. This form explains the trial in clear language. It goes over the risks and benefits, other choices for treatment, and your right to choose not to take part in the trial. You may choose to think about your choice or review the consent with family members and friends before signing the consent form. It is important to remember that if you sign the informed consent saying you want to take part in a trial, you can leave the trial at any time you want during the process.

Why should I join a trial?

There are a few reasons to take part in a clinical trial.

- In a clinical trial, you may have access to new treatments that would not otherwise be available.
- Clinical trials are important because they help the field of oncology to advance, finding safer and more helpful treatments. Today's successful treatments are because of their success in previous clinical studies.
- Unfortunately, only about 3-5% of adults with cancer take part in clinical trials, leaving many research questions unanswered. This may be due to misunderstandings about trials (from both patient and healthcare provider views), patient fears, providers not recommending a trial, or not having access to trials in their treatment center.

Clinical trials take a lot of time and money, making it hard for many places to offer them. In some cases, patients may need to

ask about clinical trials themselves to learn about them.

Phases of Clinical Trials

There are different types of oncology clinical trials:

- **Prevention** – to find ways to prevent cancer from starting.
- **Diagnostic and screening** – to find ways to find cancer early when it is easier to treat.
- **Treatment** – to find the best ways to treat cancer.
- **Supportive care** – to find ways to improve the quality of life for people getting cancer treatments.

These address all the steps in cancer care. Clinical trials look for the best care for each step that will lead to the best results for the patient. Clinical trials are done in the following phases, most often in this order, with each phase designed to answer a certain set of questions:

Phase I clinical trials:

- Try to find out the dose of a medicine (or other treatment) that is safe and to learn any side effects that the medicine may cause.
- Often involves a small number of patients, mostly with advanced disease that standard treatments have not worked for.
- Most are testing a new or novel type of treatment.
- These studies often use a dose-escalation format. In dose escalation, the first group of patients gets a certain dose, and if it seems safe and well-tolerated, the second group of patients gets a higher dose. The dose is raised for each group of patients until the maximum tolerated dose (the highest amount of medicine a person can take before it does harm).

Phase II clinical trials:

- It involves a larger number of patients and looks to test whether a treatment is effective (controls or kills cancer cells) and it keeps looking at the safety of the treatment.
- These trials also include patients who have a tumor that does not respond well to treatment. Perhaps their cancer is no longer responding to available therapies, or the type of cancer does not have any useful treatments available.
- Single-agent (1 medication) or combination regimens (multiple medicines or treatments given together) may be tested in phase II trials. The goal of these studies is to make sure that the treatment is possible, safe, and promising enough before moving on to the next phase (and a larger group of patients).

Phase III clinical trials:

- Designed to compare a study treatment to an already-used standard of care. It measures things like survival and symptom control.
- These studies are most often randomized, controlled studies (see below), done in multi-institutional settings (university and community medical centers) and involve hundreds to thousands of patients.

Phase IV clinical trials:

- Often called post-marketing studies. These studies are done once the treatment has received FDA approval and is being used in clinics. The goal of these trials is to further look at safety and effectiveness.

Can anyone take part in a trial?

Every trial is "governed" by a protocol (the trial plan). The protocol is written before the trial starts and is reviewed and approved by the IRB. It states what the goals of the trial are, which patients and how many will be included, what treatments they will get, how they will be watched, when to stop treating a patient, and so on. This document and the guidebook that the study team follows are very detailed. The protocol includes the rules (criteria) you must meet to be eligible to take part in the study. If you do not meet these strict rules, you will not be able to join.

Randomization

Phase II and III trials are often done using a technique called randomization. If you choose to take part in a randomized trial, a computer chooses the treatment you will get at random. The study team, provider, and patient have no control over this decision. In many trials, no one knows which treatment the patient will be getting (this is called blinding). This is done to fairly compare treatments, preventing the results from being affected by "bias." Bias is when human choices sway or affect a result.

In a randomized trial, there is a control group and treatment (or experimental) group.

- The control group usually gets the standard treatment for that type of cancer.
- The treatment group gets the experimental treatment being studied.
- Oncology clinical trials don't often use a placebo (a pill that does not contain any drug). If they do, it will be clearly described in the informed consent process and spelled out in the consent that is signed.

The study team can explain the design of the trial you are interested in and how it will be carried out.

Once the Trial Starts

Once in a clinical trial, your study team will help guide you through any testing that needs to be done or anything you should tell them about, such as side effects, health issues, or other concerns and questions. You should know the name of the trial and keep a copy of your consent form nearby in case you need to call your provider's office after-hours or go to an ER. If you have questions while on the trial, talk to your study team right away. If for any reason you do not want to take part in the trial any longer, it is your right to withdrawal from the study at any time.

Summary

Clinical research studies are important in improving treatment for all cancers. The treatments that we use today are a result of patients taking part in trials in the past. We are always trying to find better treatments to treat or cure cancer. There are also studies to find less toxic treatments, which can cut down on side effects. Clinical research trials are very rewarding for both the patient and the provider. The patient may benefit from new treatment options and, at the same time, help future cancer patients in their fight against this disease.

If you are interested in learning about clinical trials that may be right for you, talk with your oncology provider. You can also use the resources below to find more information about specific trials.

Clinicaltrials.gov - From the National Institute of Health, this site lists current clinical trials that are open to new participants who meet specific trial qualifications.

<http://www.clinicaltrials.gov>

Emerging Med Navigator - Provides telephone and online help for finding clinical trials.

<https://app.emergingmed.com/OncoLink/home>

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